
In Utero Hematopoietic Stem Cell Transplantation For The Treatment Of Fetuses With Alpha Thalassemia Major

Grant Award Details

In Utero Hematopoietic Stem Cell Transplantation For The Treatment Of Fetuses With Alpha Thalassemia Major

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-09183

Project Objective: To establish the safety of in utero transplantation in fetuses with alpha thalassemia major.

Investigator:

Name:	Tippi MacKenzie
Institution:	University of California, San Francisco
Type:	PI

Disease Focus: Blood Disorders, Fetal Alpha Thalassemia Major

Human Stem Cell Use: Adult Stem Cell

Award Value: \$10,906,978

Status: Active

Grant Application Details

Application Title: IN UTERO HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR THE TREATMENT OF FETUSES WITH ALPHA THALASSEMIA MAJOR

Public Abstract:**Therapeutic Candidate or Device**

Maternal bone marrow-derived CD34+ hematopoietic stem cells.

Indication

Fetal alpha thalassemia major.

Therapeutic Mechanism

This strategy that takes advantage of existing tolerance between the mother and fetus during pregnancy, so that maternal cells can be transplanted into a fetus without conditioning or immunosuppression. Survivors of alpha thalassemia need chronic blood transfusions or a stem cell transplant after birth, both of which have significant morbidity; if successful, in utero transplantation could result in a definitive cure, or allow postnatal boost transplant with decreased morbidity.

Unmet Medical Need

Alpha thalassemia major is almost always fatal in utero, and rare survivors need costly and morbid chronic care. There is an unmet clinical need to develop a therapy that would be life-saving, yet avoid the chronic disease burden of current survivors.

Project Objective

Phase 1 trial completed.

Major Proposed Activities

- Manufacture maternal bone marrow stem cells.
- Establish the safety of in utero transplantation in fetuses with ATM
- Establish the feasibility of maternal bone marrow harvest and in utero transplantation.

Statement of Benefit to California:

Fetal surgery was invented in California and we continue to lead the field in developing novel in utero therapies. The incidence of the target disease, ATM, is highest in California due to immigration patterns. Most importantly, successful completion of this trial will open the door for applications of in utero stem cell transplantation in other common blood disorders such as sickle cell disease (California incidence: 15.2/100,000 births), and beta thalassemia (1.8/100,000 births).

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